
CIRM Board Approves New Clinical Trial for ALS

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Oakland, CA – Today the governing Board of the California Institute for Regenerative Medicine (CIRM) awarded \$11.99 million to Cedars-Sinai to fund a clinical trial for amyotrophic lateral sclerosis (ALS), also known as Lou Gehrig's disease.

ALS is a neurodegenerative disease that results in the death of nerve cells in the brain and spinal cord, causing the muscles in the body to gradually weaken, leading to loss of limb function, difficulty breathing, paralysis, and eventually death. There are medications that can slow down the progression of ALS, but unfortunately there is no cure for the disease.

Clive Svendsen, Ph.D., executive director of Cedars-Sinai's Board of Governors Regenerative Medicine Institute, and his team will be conducting a trial that uses a combined cell and gene therapy approach as a treatment for ALS. The trial builds upon the Stem Cell Agency's first ALS trial, also conducted by Cedars-Sinai and Svendsen.

Genetically engineered stem cells will be transplanted into the motor cortex, an area of the brain responsible for voluntary movements. These transplanted cells then become astrocytes, a type of support cell that help keep nerve cells functioning. The astrocytes have been genetically altered to deliver high doses of a growth factor which has been shown to protect nerve cells. The goal of this approach is to protect the upper motor neurons controlling muscle function and meaningfully improve the quality of life for ALS patients.

"ALS is a devastating disease that attacks the spinal cord and brain and results in the progressive loss of the ability to move, to swallow and eventually to breathe." says Maria T. Millan, M.D., President and CEO of CIRM. "This clinical trial builds on Dr. Svendsen's work previously funded by CIRM. We are fortunate to be able to support this important work, which was made possible by California citizens who voted to reauthorize CIRM under Proposition 14 this past November."

About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With \$5.5 billion in funding and more than 150 active stem cell programs in our portfolio, CIRM is the world's largest institution dedicated to helping people by bringing the future of cellular medicine closer to reality.

For more information go to www.cirm.ca.gov

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